Genome Editing Techniques and Their Therapeutic Applications.

Journal: Clin Pharmacol Ther

Publication Year: 2017

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PubMed link: 27783398

Funding Grants: Engineered iPSC for therapy of limb girdle muscular dystrophy type 2B

Public Summary:
This review article draws on my decades of experience in the genetic engineering field. The article summarizes most of the currently used methods to modify the human genome, stressing how these methods can be used in gene and cell therapy strategies. In particular, the article explains the basis and evolution of editing methods that use a double-strand break, including meganucleases, zinc finger nucleases, TALENs, and the current CRISPR/Cas type systems. In addition, older methods that continue to be valuable are featured, including retrovirus, lentivirus, transposon, and site-specific recombinase approaches. Overall, the article provides a comprehensive overview of gene editing methods, how they evolved, and how they can be used in therapeutic strategies.

Scientific Abstract:
Fueled by advances in the field of genetics, the methods available to edit DNA sequences in living cells have continued to develop steadily. These technologies directly impact the fields of gene and cell therapy, where changes in the DNA sequence of target cells offer a route to correct genetic diseases and manipulate disorders like cancer. We review here the expanding menu of genome editing techniques and how they are being applied to therapeutic targets. The methods encompass a myriad of approaches to modify the covalent structure of DNA, including the targeted creation of double-strand breaks that can catalyze genomic changes, as well as the use of retroviruses and transposons to mediate gene addition, recombinases for sequence-specific gene addition and deletion, and base repair for direct sequence changes. The continued growth of the exciting field of genome editing is opening new possibilities for therapeutic intervention.